

Biological Therapeutics for Rare Plasma Protein Disorders Workshop

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Meeting Objectives and Overview

Overall objective is to facilitate the development of products to treat patients with very rare plasma protein disorders.

- Learn about the need for, and current availability of these products
- Identify challenges to product development
- Review current product development procedures and experience from the perspectives of regulators and sponsors
- Identify opportunities to facilitate clinical trials
- Suggest new ideas for product development

Meeting Agenda – Day 1

- Current challenges in the availability and development of biological products to treat rare plasma protein disorders
 - Perspectives of patients and physicians
 - International perspective: what is the scope of the patient population, and what products are available in other countries but not the US?
 - Discuss factors that affect industry's ability to bring new biotherapies to patients
 - FDA's historical experience in reviewing products for very small populations
 - Open Public Discussion

Meeting Agenda – Day 1

- Current Opportunities: What are the current regulatory pathways and incentives to develop biological products for very small populations?
 - International perspective : European Medicinal Authority (EMA)
 - FDA perspective on clinical trial design for very small populations
 - Discussion of the FDA accelerated approval process
 - Statistical considerations for very small clinical trials
 - Orphan Drug provisions and incentives
 - Open panel discussion

Meeting Agenda – Day 1

- Research support from the NHLBI for rare plasma protein disorders
- Example of NHLBI support through the Small Business Innovative Research Support grant
- Review of Medicare payment program
- Open discussion of these initiatives

Meeting Agenda – Day 2

- Case Studies of product development:
 - Protein C,
 - Factor XIII,
 - Antithrombin III,
 - Treatment of Platelet disorder
 - Treatment for Fabray's disease
- Open discussion

Meeting Agenda – Day 2

- Future opportunities: Enhanced Data Collection
 - FDA and EMEA experience with post marketing data collection
 - Experience of sponsors in collecting postmarketing surveillance data through third parties
 - Consumer group-initiated post marketing surveillance
 - Opportunities for data collection through registries and the CDC
 - Open Discussion
- Final panel discussion: where do we go from here?